MeiraGTx Reports Fourth Quarter and Full Year 2022 Financial and Operational Results

March 14, 2023

- *Announced positive clinical data from AQUAx AAV-hAQP1 Phase 1 trial for treatment of Grade 2/3 Radiation-Induced Xerostomia in December 2022*

- *Announced positive top-line data from the MGT009 Phase 1/2 clinical study demonstrating safety and improvement in multiple domains of vision in X-linked retinitis pigmentosa (XLRP) patients treated with botaretigene sparoparvovec (AAV-RPGR) compared to untreated randomized control at the American Academy of Ophthalmology (AAO) 2022 Annual Meeting*

- *Continued enrollment and dosing of patients in pivotal Phase 3 Lumeos clinical trial of botaretigene sparoparvovec for the treatment of XLRP*

- *On track for BLA submission of botaretigene sparoparvovec for the treatment of XLRP in 2024*

LONDON and NEW YORK, March 14, 2023 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced financial and operational results for the fourth quarter and full-year ended December 31, 2022, and provided a corporate update.

“As we begin 2023, we are increasingly confident in all three of our lead clinical programs, as well as our transformative riboswitch gene regulation technology,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “In the fourth quarter of last year, we announced positive clinical data from our Phase 1 trial of AAV-hAQP1 for the treatment of grade 2/3 radiation-induced xerostomia and presented positive data at AAO demonstrating positive safety and sustained vision improvement in patients with X-linked retinitis pigmentosa who were treated with botaretigene sparoparvovec in our Phase 1/2 trial. We also presented 15 abstracts at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress highlighting just some of the data from our gene control platforms, including for the first time our riboswitch gene regulation applied to cell therapy. In addition, we began dosing patients with adeno-associated virus (AAV) encoding glutamic acid decarboxylase (AAV-GAD), an investigational gene therapy for Parkinson’s disease.”

Dr. Forbes continued, “This year, in XLRP, we intend to complete enrollment of the pivotal Lumeos Phase 3 study and are on track for a BLA filing in 2024. In xerostomia, we intend to begin a randomized, double-blind, placebo-controlled, Phase 2 study in the second quarter, and in AAV-GAD for Parkinson’s, we anticipate completing enrollment of our current study in the second half of 2023 as we move forward with discussions with global regulators regarding a path to BLA. This progress across multiple programs and platforms is enabled by our broad end-to-end capabilities in vectorology optimization and manufacturing. In addition, in 2023, we intend to share more about our plans to use our riboswitch technology to allow gene therapy to be applied to more prevalent diseases in a cost-effective way. This has been one of our primary goals since the inception of MeiraGTx, and we are very excited to be advancing towards potentially achieving this ambition.”

Recent Development Highlights and Anticipated 2023 Milestones

Botaretigene Sparoparvovec for the Treatment of XLRP:

- On October 1, 2022, clinical data from a Phase 1/2 MGT009 clinical trial (NCT03252847) were presented in a late-breaking oral presentation at the Retina Subspecialty Day program of the AAO 2022 Annual Meeting; treatment with botaretigene sparoparvovec was found to have an acceptable safety profile and efficacy assessments in this study and demonstrated improvements in retinal sensitivity, visual function and functional vision.[1]
- Further sensitivity analysis was conducted on study participants by applying the Phase 3 LUMEOS (NCT04671433) study eligibility criteria that corroborated the endpoints selected for the Phase 3 study.[1]
- MeiraGTx, in collaboration with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, is dosing patients in the pivotal Phase 3 LUMEOS clinical trial of botaretigene sparoparvovec and remains on track for a BLA submission in 2024.

AAV-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia:

- MeiraGTx reported positive clinical data from the AQUAx Phase 1 clinical trial in December 2022.
  - Clinically meaningful improvements in xerostomia symptoms and disease burden in two validated Patient-Reported Outcome (PRO) measures in both unilateral and bilateral treated cohorts were demonstrated.
    - 18/24, or 75% achieved clinically meaningful symptom improvement using the Global Rate of Change (GRCQ) PRO.
    - Using the Xerostomia Questionnaire (XQ), 71% (17/24) reported an improvement of >8 points (clinically meaningful), and 67% (16/24) had an improvement of ≥10 (considered transformative by KOLs).
  - Meaningful increases in whole saliva flow rates were observed post-treatment, providing objective evidence of the biological activity of AAV-hAQP1 treatment.
  - Early long-term follow-up data suggest durability of improvement 2+ years post-treatment.

    - AAV-hAQP1 appears safe and well-tolerated at each dose tested.
- All participants are followed for 1 year post-treatment and then enter a long-term follow-up study for another 4 years.
- The Company intends to present the final 12 month data from the bilateral treated cohorts from the AQUAx Phase 1 study in the second quarter of 2023.
- Based on the favorable safety and efficacy profile of AAV-hAQP1 in the AQUAx Phase 1 study, the Company intends to initiate a randomized, double-blind, placebo-controlled, Phase 2 study evaluating the bilateral administration of two active doses of AAV-hAQP1 in the second quarter of 2023.

AAV-GAD for the Treatment of Parkinson's Disease :

- The Company is now dosing patients in the AAV-GAD clinical trial under a new IND using material manufactured in its cGMP facility in London, United Kingdom using MeiraGTx's proprietary production process.
- The AAV-GAD trial is a three-arm randomized Phase 1 clinical bridging study with subjects randomized to one of two doses of AAV-GAD or sham control.
- The objective of the AAV-GAD trial (NCT05603312) is to evaluate the safety and tolerability of AAV-mediated delivery of glutamic acid decarboxylase (GAD) gene transfer into the subthalamic nuclei (STN) of participants with Parkinson's disease.
- Completion of enrollment is anticipated by the third quarter of 2023.

Riboswitch Gene Regulation Platform & Vector Engineering:

- The Company exhibited 15 poster presentations at the ESGCT 2022 Annual Congress, which included data from MeiraGTx's novel gene regulation platform, including the first data demonstrating the potential to regulate cell therapies including CAR-T, as well as data from the Company's promoter platforms and several new, optimized pre-clinical programs addressing severe unmet needs for indications such as amyotrophic lateral sclerosis (ALS) and Wilson's disease. In addition, the Company made several presentations on its proprietary viral vector manufacturing technology and potency assay development.
- The Company's next-generation riboswitch-based gene regulation platform can be used to precisely control the expression of any gene delivered in any context with an unprecedented dynamic range using novel, synthetic, orally delivered small molecules.
- The Company now has over 30 novel orally available small molecules with high specificity and potency to its riboswitch aptamers moving through PK, biodistribution and toxicology studies, with the first GMP material for IND currently being manufactured.

Gene Therapy Manufacturing:

- MeiraGTx's wholly-owned facilities have now produced GMP clinical trial material for 6 different indications, using multiple AAV serotypes, including administration into the eye, salivary gland and central nervous system.
- The Company believes that its proprietary platform production process has produced one of the highest yields and full ratios in the industry.
- The Company believes that bringing all aspects of testing and vector production in-house reduces regulatory risk, ensures the highest quality of products, lowers costs and helps avoid bottlenecks in clinical development.
- In addition to its 30,000-square-foot facility in London, MeiraGTx now has a 150,000-square-foot plant in Shannon, Ireland which contains three facilities: one built to be flexible and scalable for viral vector production, another to manufacture plasmid DNA – the critical starting material for producing gene therapy products – and third, a Quality Control (QC) hub performing advanced biochemical quality control testing appropriate for commercialization.

For more information related to our clinical trials, please visit www.clinicaltrials.gov

As of December 31, 2022, MeiraGTx had cash and cash equivalents of approximately $115 million, as well as approximately $21 million in receivables due from Janssen from the fourth quarter of 2022. The Company believes that with such funds, as well as anticipated milestones from Janssen, it will have sufficient capital to fund operating expenses and capital expenditure requirements into the fourth quarter of 2024.

Financial Results

Cash and cash equivalents were $115.5 million as of December 31, 2022, compared to $137.7 million as of December 31, 2021.

License revenue was $15.9 million for the year ended December 31, 2022, compared to $37.7 million for the year ended December 31, 2021. This decrease is a result of MeiraGTx receiving a $30.0 million milestone payment in connection with the Janssen collaboration during the year ended December 31, 2021.

General and administrative expenses were $46.6 million for the year ended December 31, 2022, compared to $43.8 million for the year ended December 31, 2021. The increase of $2.8 million was primarily due to an increase in share-based compensation, legal and accounting fees, consulting fees and depreciation, which was partially offset by decreases in payroll and payroll-related costs, insurance, rent and facilities costs and other general and administrative expenses.

Research and development expenses for the years ended December 31, 2022, and 2021 were as follows (in millions):

	2022	2021	Change
Gross research and development expenses	$ 165.8	$ 141.1	$ 24.7
Janssen reimbursements	(73.3)	(69.0)	(4.3)
Tax incentive reimbursement	(6.8)	(5.4)	(1.4)
Research and development expenses	$ 85.7	$ 66.7	$ 19.0

Gross research and development expenses for the year ended December 31, 2022, increased $24.7 million as compared to the prior year primarily due to an increase in costs related to manufacturing of our clinical trial materials, payroll and payroll-related costs, costs related to our pre-clinical research and clinical trials, share-based compensation, rent and facility costs, depreciation and other research costs, which was partially offset by a decrease in license fees and acquired research and development costs.

Reimbursements under the Janssen collaboration agreement for the year ended December 31, 2022, increased $4.3 million as compared to the prior year primarily due to an increase in activity in the programs licensed under the Janssen collaboration agreement.

Tax incentive reimbursement for the year ended December 31, 2022, increased $1.4 million as compared to the prior year primarily due to the increase in allowable research and development costs.

Foreign currency loss was $9.5 million for the year ended December 31, 2022, compared to a loss of $6.3 million for the year ended December 31, 2021. The increase in the loss of $3.2 million was primarily due to an unrealized loss on the valuation of the Company's intercompany payables and receivables due to the strengthening of the U.S. dollar against the pound sterling and euro during the year ended December 31, 2022.

Interest income was $0.8 million for the year ended December 31, 2022, compared to $0.2 million for the year ended December 31, 2021. The increase was due to a higher interest rate during 2022.

Interest expense was $4.9 million for the year ended December 31, 2022, compared to $0.3 million for the year ended December 31, 2021. The increase was primarily due to the interest on the Company's outstanding debt.

Net loss attributable to ordinary shareholders for the year ended December 31, 2022, was $129.6 million, or $2.87 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $79.6 million, or $1.80 basic and diluted net loss per ordinary share for the year ended December 31, 2021.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage gene therapy company with six programs in clinical development and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, and a transformative gene regulation platform technology that allows precise, dose responsive control of gene expression by oral small molecules with dynamic range that can exceed 5000-fold. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: ocular diseases, including both inherited retinal diseases as well as large degenerative ocular diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system and salivary gland, MeiraGTx plans to expand its focus to develop additional gene therapy treatments for patients suffering from a range of serious diseases.
For more information, please visit www.meiragtx.com

Forward Looking Statement

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding our product candidate development and anticipated milestones regarding our pre-clinical and clinical data, reporting of such data and the timing of results of data and regulatory matters, including in light of the COVID-19 pandemic, as well as statements that include the words "expect," "will," "intend," "plan," "believe," "project," "forecast," "estimate," "may," "could," "should," "would," "continue," "anticipate" and similar statements of a future or forward-looking nature. These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, raise additional capital, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of the COVID-19 pandemic on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2022, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC's website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

[1] Michaelides, M et al. Ph1/2 AAV5-RPGR (Botaretigene Sparoparvovec) Gene Therapy Trial in RPGR-associated X-linked Retinitis Pigmentosa (XLRP). Abstract #30071754. Presented at the 2022 American Academy of Ophthalmology Annual Meeting.

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MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)

	For the Years Ended December 31,	
	2022	2021
License revenue - related party	$ 15,920	$ 37,701
Operating expenses:		
General and administrative	46,550	43,765
Research and development	85,725	66,694
Total operating expenses	132,275	110,459
Loss from operations	(116,355)	(72,758)
Other non-operating income (expense):		
Foreign currency loss	(9,452)	(6,293)
Interest income	777	212
Interest expense	(4,946)	(288)
Fair value adjustments	361	(434)
Net loss	(129,615)	(79,561)
Other comprehensive income:		
Foreign currency translation gain	8,718	2,226
Comprehensive loss	$ (120,897)	$ (77,335)
Net loss	$ (129,615)	$ (79,561)
Basic and diluted net loss per ordinary share	$ (2.87)	$ (1.80)
Weighted-average number of ordinary shares outstanding	45,177,857	44,139,655

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share amounts)

	December 31, 2022	December 31, 2021
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 115,516	$ 137,703
Accounts receivable - related party	21,334	22,384
Prepaid expenses	8,133	8,102
Tax incentive receivable	7,689	12,634
Other current assets	1,667	2,420

Total Current Assets	154,339	183,243
Property, plant and equipment, net	109,266	75,860
Intangible assets, net	1,335	1,791
In-process research and development	742	783
Other assets	1,402	1,404
Equity method and other investments	6,326	6,656
Right-of-use assets - operating leases, net	20,109	22,782
Right-of-use assets - finance leases, net	24,718	27,645
TOTAL ASSETS	$ 318,237	$ 320,164
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 16,616	$ 15,348
Accrued expenses	39,818	27,586
Lease obligations, current	3,884	3,374
Deferred revenue - related party, current	15,123	21,820
Other current liabilities	6,631	—
Total Current Liabilities	82,072	68,128
Deferred revenue - related party	27,436	43,046
Lease obligations	17,331	20,359
Asset retirement obligations	2,179	2,081
Deferred income tax liability	186	196
Note payable, net	71,033	—
Other long-term liabilities	262	953
TOTAL LIABILITIES	200,499	134,763
COMMITMENTS AND CONTINGENCIES (Note 15)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 48,477,209 and 44,548,925 shares issued and outstanding at December 31, 2022 and 2021, respectively	2	2
Capital in excess of par value	581,893	528,659
Accumulated other comprehensive income (loss)	6,047	(2,671)
Accumulated deficit	(470,204)	(340,589)
Total Shareholders' Equity	117,738	185,401
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 318,237	$ 320,164